Application Number: T2-00005 **Scientific Score**: 81

Title: Stem Cell Training Grant

Specific names of individuals and institutions are blacked out to preserve applicant confidentiality where possible.

Proposal Abstract as Submitted by Applicant

This Level II Training Grant will support seven PhD Post-Doctoral and three MD Clinical Fellows at [redacted] for training in stem cell biology, and the clinical and ethical implications of stem cell research. [redacted] research has been consistently ranked in the top six of the nation's pediatric stand alone institutions by the NIH. Over the past 20 years, [redacted] has built an internationally renowned research program in stem cell biology and its clinical applications. The program was founded on the fields of human hematopoietic stem cell biology, transplantation and gene therapy. In the past decade, the program has been expanded to include somatic stem cells from lung, pancreas, liver, gut, bladder and mesenchyme. In the past three years, investigators at [redacted] have developed expertise in human embryonic stem cells (hESC) and established a hESC training and tissue culture core for [redacted] investigators. A unique focus of the Training Program at [redacted] will be the application to pediatric disorders such as diabetes, monogenic inherited disorders (cystic fibrosis, muscular dystrophy, sickle cell disease, etc), and congenital birth defects. It is our central hypothesis that childhood disorders will be especially responsive to therapies produced by the use of stem cells; advances in the use of stem cells to treat childhood illnesses will then lead the way to treatments for the many disorders that occur later in life. The training program includes: a didactic course on "Stem Cell Biology, Research Methods and Stem Cell Therapies", a course titled "The Ethics of Stem Cell Research and Therapies", participation in multiple existing training activities at [redacted], training in laboratory methods in hESC and joint participation in a didactic course taught by scientists from [redacted], [redacted] and the [redacted]. A Steering Committee with stem cell researchers, clinical physician/scientists and medical educators will oversee selection and supervision of trainees, the mentoring process, and other activities of the training program. The biomedical environment and strength of stem cell research at [redacted] combine to provide a rich milieu for training the next generation of physicians and scientists who will use stem cells as the basis for research and therapy.

Benefit of this Program to California

This program will benefit the people and the state of California by providing high-quality training in the scientific, clinical, social, and ethical aspects of stem cell research to the scientists and clinicians who will develop and apply future therapies in this rapidly emerging field.

Summary of Review

This is an extremely well written and clearly presented type II proposal that focuses training of post-doctoral and clinical fellows in the basic science, clinical, and ethical aspects of stem cells. This institution has built an internationally renowned research

program in stem cell biology and has recently developed expertise in human embryonic stem cells. A unique focus of the training program will be in the application to pediatric disorders such as diabetes, cystic fibrosis, and congenital birth defects. The program builds on existing training programs and offers courses in methods and therapies as well as ethical, legal, and social implications of stem cell research. The support activities are very well presented including an existing monthly meeting of a stem cell interest group, which has met since 2003 and where data, new experimental approaches, and ideas are shared. The program director is a well-respected clinical investigator and experienced administrator whose research focuses on cord-blood stem cells. Internal and external steering committees will offer solid quality control and evaluation of the training program, although it is not clear how linkages will be established and maintained between clinical and basic science departments. Proposed mentors include a large number of junior faculty members with average to good publication records, many in stem cell-related areas. The proposal describes a selection process that includes minority students. Although it is difficult to ascertain where the applicants will come from, past trainees of the institution demonstrate a diverse ethnic distribution. The institution is a solid and respected training environment and offers excellent core facilities for hESC research and stem cell experiments in mice.

Overall Strengths and Weaknesses

This strong application presents a training program for post-doctoral and clinical fellows. It exploits the excellent facilities and clinical science strength of this institution, particularly in the application of stem cell research to pediatric disease. It builds on an existing training program and adds new courses and support activities that provides a good mixture of basic research and clinical application. The somewhat unimpressive publication record of the largely junior training faculty is considered a weakness of the program.

Recommendations

Highly meritorious and recommended for funding.

	Pre	Post	Clinical	Total
Fellows Requested:	0	7	3	10
Fellows Recommended:	0	7	3	10

	Year 1	Total
Budget Requested:	$ 796,942	$ 2,390,826
Budget Recommended:	$ 796,942	$ 2,390,826